

Rethinking Venture Philanthropy After the Kalydeco Windfall

Peter Kolchinsky | 12/22/14

The Cystic Fibrosis (CF) Foundation's big win in venture philanthropy can fuel constructive competition among companies developing innovative CF drugs, benefiting both patients and the healthcare system by increasing future treatment options and reducing their cost.

CF is a fatal genetic disease affecting around 30,000 people in the U.S. that is caused by mutations in the cystic fibrosis transmembrane receptor (CFTR) gene. These mutations disrupt either the expression or function of the CFTR protein, causing mucus buildup in the lungs that can impair breathing and lead to infection. Although the most severe symptoms of CF impact the lungs, the disease also leads to a shortage of the pancreatic enzymes needed for digestion.

The vast majority of drugs marketed to treat CF address the symptoms, and not the cause of the disease. Ivacaftor (Kalydeco), a drug from Vertex Pharmaceuticals (NASDAQ: VRTX) that was developed with an investment from the CF Foundation, is the only therapy available that addresses the underlying cause of CF, though currently only for a small fraction of patients with particular mutations. Vertex is developing other drugs, so-called CFTR correctors, that can be combined with ivacaftor to address more CF patients.

Royalty Pharma's \$3.3 billion purchase of the CF Foundation's roughly 10 percent royalty on Vertex's CF drugs last month sparked some controversy. Articles in the New York Times and Xconomy suggested that the foundation had somehow failed patients by allowing Vertex to price its drugs so high that a 10 percent royalty could be worth so much. These criticisms echo those directed at the foundation when ivacaftor hit the market in 2012.

Ivacaftor's price tag, about \$300,000 per year, per patient, shocked the market. Critics declared that the CF Foundation should have done more to ensure an affordable price for patients. They further insinuated that the drug's price was evidence that the foundation had a conflict of interest; it could not simultaneously serve patients and fund biotech companies. In the wake of the multibillion-dollar royalty sale, critics

are now repeating these same accusations.

These critics are missing an important part of the CF Foundation's strategy. While the foundation could not possibly have any leverage over how Vertex priced its drug, by harvesting \$3.3 billion now, it will be able to speed the development of over a dozen early competitors. This will usher in an era of competition that will help make the CF therapies of the near future not only better, but also less expensive—long before Vertex's drugs go generic.

Because ivacaftor is the first drug on the market to treat the root cause of CF, Vertex has a monopoly. The company can set prices based on its own internal calculations without competitive pressure from companies with other therapeutic options. In the original agreement between the CF Foundation and Vertex, the foundation was to collect royalties on drug sales over the 15 or more years that Vertex's drugs are protected by patents. The deal with Royalty Pharma gives the foundation a large sum of money today that it can immediately invest in the programs of Vertex's competitors, speeding their drugs to market. Prior to this royalty transaction, it had already provided funding to other companies with rival CF drugs in clinical trials that could similarly address the underlying cause of CF, sowing the seeds for a more competitive future market. The price Royalty Pharma paid for the Vertex royalty, at least compared to value of Vertex itself (some \$27 billion), suggests that some price erosion for Vertex's CF franchise was already factored into the deal. We estimate that Vertex retained about 55 percent of its drugs' after-tax profits, while Royalty Pharma bought 10 percent. Since Vertex is currently valued almost entirely on the basis of its CF franchise, then 55 percent of the CF franchise is worth \$27 billion. Ten percent should be worth just under \$5 billion, but Royalty Pharma paid \$3.3 billion, a third less. While this math is only approximate and quite simplified, a discount of this magnitude might indicate that Royalty Pharma expects price erosion from the competition the CF Foundation would empower with its windfall.

To preempt competition that would help drive prices down, Vertex could acquire many of its smaller, would-be rivals, ensuring its long-term dominance in CF. Five years ago when Vertex was the dominant player in the hepatitis C market, it did not pursue this strategy. Vertex has since been swept from the field by better, second-to-market drugs. Vertex may have learned from this experience, and might be more aggressive about protecting its CF franchise. If the company is able to put this strategy into effect, the quality of Vertex's CF drugs would likely improve further. What's more, that franchise would stay at a very high price over the long run; the cost is actually borne primarily by the healthcare system (i.e., all of us) as a whole, and not by patients, who are insulated from the true costs of specialty pharmaceuticals through the use of co-pay assistance programs.

The chance that Vertex might consolidate the competitive landscape makes it all the more interesting that the CF Foundation has converted its royalty stream into a war chest with which to arm the competition, though the foundation has called it "reinvesting to accelerate further drug discovery." Regardless of how this development is communicated, the foundation's creativity on behalf of patients and the entire healthcare system should be recognized. It is now able to help many of the smaller companies generate clinical data that would allow them to be appreciated and ultimately funded by investors. Those companies could even end up being acquired by big players like AbbVie or Novartis, which have CF drugs in their pipelines and are looking to compete with Vertex.

While pharma stalwarts would claim that fighting on price is not how innovative drugs are differentiated, price wars are already a reality and, in certain situations, inevitable. For example, in the type 2 diabetes market, Merck's sitagliptin (Januvia) was the first DPP-4 inhibitor to reach the market in 2006. Although in the U.S., sitagliptin enjoyed three years of market exclusivity before the first competing drug was approved, the onset of competition upended Merck's position and drove down the price of sitagliptin through more aggressive rebates and discounts. When there are several good options and payers care about the magnitude of the expense, price wars are a likely outcome.

We should remember that the CF Foundation helped catalyze a major breakthrough in the treatment of CF. By retaining and monetizing a royalty on that first big win, the foundation now has a chance to help bring about other treatment options and the competitive pricing pressure that would come with them—providing even more benefits to patients and the healthcare system. The CF Foundation should be commended for its forward-thinking strategy and all it has done for patients and families with CF. Hopefully, non-profits serving other patient groups will borrow from its playbook.

—Peter Kolchinsky is RA Capital's founder, Managing Director, and Portfolio Manager.

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